ELA families-scientists meeting 2023
Scientific Program

Day 1 - Saturday April 15th

Introduction Guy Alba, *president of ELA International* 2 PM CET

Adrenoleukodystrophy ALD - Adrenomyeloneuropathy AMN 2:15 PM

Isabelle WEINHOFER (Austria): A blood test to track brain damage: Biomarker-based risk prediction for the onset of cerebral ALD

Lisa SCHAFER (Germany): Clinical symptoms and quality of life in women with adrenoleukodystrophy

Florian EICHLER (USA): Treatments for Adults with ALD: One Size Does Not Fit All

Wolfgang KOEHLER (Germany): Lessons learned from a first International controlled clinical trial with Leriglitazone in men with Adrenomyeloneuropathy

Elise YAZBECK (France): Update on the Minoryx clinical trial in childhood cerebral adrenoleukodystrophy

Questions & Answers

BREAK **4:25 PM**

Genetic and undetermined leukodystrophies - Pelizaeus-Merzbacher Disease PMD - POLR3-HLD 4H 4:40 PM

Laura ADANG (USA): Diagnosing and management of undiagnosed leukodystrophies

Nicole WOLF (The Netherlands): Hypomyelination – what's new?

Vivi HEINE (The Netherlands): Cortical interneuron involvement in 4H leukodystrophy

Noémie HAMILTON (UK): Modelling RNASET2 leukodystrophy in zebrafish to develop transformative therapies

Questions & Answers

END of day 1 **6:50 PM**

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Day 2 - Sunday April 16th

Introduction Guy Alba, *president of ELA International* 2 PM CET

Megalencephalic leukoencephalopathy with subcortical cysts MLC, CACH syndrome VWM, Canavan and Alexander diseases 2:15 PM

Elena AMBROSINI (Italy): Molecular defects in MLC disease: how basic research can help finding therapeutic strategies

Marjo van der KNAAP (The Netherlands): Update on vanishing white matter VWM - CACH syndrome

Matthias ECKHARDT (Germany): Does the neuropeptide NAAG plays a role in the pathogenesis of Canavan disease?

Angela GRITTI & Vasco MENEGHINI (Italy): Development of editing technologies to treat Alexander disease

Milos PEKNY (Sweden): Alexander disease - the road ahead

Questions & Answers

BREAK **4:25 PM**

Aicardi-Goutières Syndrome AGS, Zellweger spectrum disorders, Krabbe disease and Metachromatic leukodystrophy MLD 4:40 PM

Marie-Louise Frémond (France): Follow-up of an ELA-funded trial in Aicardi-Goutières syndrome

Femke KLOUWER (The Netherlands): Zellweger spectrum disorders

Marco CECCHINI & Ambra DEL GROSSO (Italy): Nanomedicine and Autophagy modulation in the mouse model of Krabbe disease

Caroline SEVIN (France): Management and treatment options in metachromatic leukodystrophy and Krabbe disease

Questions & Answers

END **6:50 PM**